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Substitute for form 1449/PTO

INFORMATION DISCLOSURE STATEMENT BY APPLICANT

(Use as many sheets as necessary)

Complete if Known

Application Number	10/586,892				
Filing Date	February 9, 2007				
First Named Inventor	David S. Lawrence				
Art Unit	1636				
Examiner Name	Jennifer Ann Dunston				
Sheet	1	of	3	Attorney Docket Number	96700/1160

NON PATENT LITERATURE DOCUMENTS

Examiner Initials*	Cite No. ¹	Include name of the author (in CAPITAL LETTERS), title of the article (when appropriate), title of the item (book, magazine, journal, serial, symposium, catalog, etc.), date, page(s), volume-issue number(s), publisher, city and/or country where published.	T ²
	1	BOOMS P et al. "Novel exon skipping mutation in the fibrillin-1 gene: two 'hot spots' for the neonatal Marfan syndrome." Clin. Genet. 55:110-7, 1999. Abstract only	
	2	DU M et al. PTC124 is an orally bioavailable compound that promotes suppression of the human CFTR-G542X nonsense allele in a CF mouse model. PNAS 105: 2064-69, 2.	
	3	DURIEZ B et al. "An exon-skipping mutation in the btk gene of a patient with X-linked agammaglobulinemia and isolated growth hormone deficiency." FEBS Lett. 1994	
		June 13;346(2-3):165-70. Abstract only	
	4	ENZMANN H et al. Damage to mitochondrial DNA induced by the quinolone Bay y 3118 in embryonic turkey liver. Mutat. Res. 425:213-24, 1999. Abstract only	
	5	FERNANDES R et al. Incorporation of structurally defective type II collagen into cartilage matrix in kniest chondrodysplasia. Arch Biochem Biophys 355:282-90 1998 Abstract	
	6	FOGLI A et al. "Intracellular levels of the LIS1 protein correlate with clinical and neuroradiological findings in patients with classical lissencephaly."	
		Ann Neurol. 1999 Feb;45(2):154-61. Abstract only	
	7	GUILFORD P et al. "E-cadherin germline mutations in familial gastric cancer." Nature 1998 Mar. 26;392(6674):402-5. Abstract only	
	8	HIRANO M et al. Dominant negative effect of GTP cyclohydrolase I mutations in dopa-responsive hereditary progressive dystonia. Ann Neurol. 44:365-71 1998 Abstract	

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¹ Applicant's unique citation designation number (optional). ² Applicant is to place a check mark here if English language Translation is attached.

This collection of information is required by 37 CFR 1.98. The information is required to obtain or retain a benefit by the public which is to file (and by the USPTO to process) an application. Confidentiality is governed by 35 U.S.C. 122 and 37 CFR 1.14. This collection is estimated to take 2 hours to complete, including gathering, preparing, and submitting the completed application form to the USPTO. Time will vary depending upon the individual case. Any comments on the amount of time you require to complete this form and/or suggestions for reducing this burden, should be sent to the Chief Information Officer, U.S. Patent and Trademark Office, P.O. Box 1450, Alexandria, VA 22313-1450. DO NOT SEND FEES OR COMPLETED FORMS TO THIS ADDRESS. SEND TO: Commissioner for Patents, P.O. Box 1450, Alexandria, VA 22313-1450.

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	9	HIRAWAT S et al. Safety, tolerability, and pharmacokinetics of PTC124, a nonaminoglycoside nonsense mutation suppressor, following single- and multiple-dose administration to			
		healthy male and female adult volunteers. Clin. Pharm. 2007; 47:430-444.			
	10	KEREM E et al. Effectiveness of PTC124 treatment of cystic fibrosis caused by nonsense mutations: a prospective phase II trial. The Lancet vol. 372: 719-27, 2008.			
	11	LAUMONNIER Fet al. X-Linked mental retardation and autism are associated with a mutation in the NLGN4 gene, a member of the neuroligin family. Am J Hum. Genet. 74:552-7, 2004.			
	12	MACOSKA J et al. "Loss of expression of human spectrin Src homology domain binding protein 1 is associated with 10p loss in human prostatic adenocarcinoma."			
		Neoplasia, 3:99-104, 2001.			
	13	MERCURI E et al. "Muscle MRI findings in a three-generation family affected by Bethlem myopathy." Eur. J. Paediatr. Neurol. 2002;6(6):309-14. Abstract only			
	14	NICHOLLS A et al. "An exon skipping mutation of a type V collagen gene (COL5A1) in Ehlers-Danlos syndrome." J. Med. Genet. 1996;33:940-946.			
	15	THOMAS A et al. "Electrochemical characteristics of five quinolone drugs and their effect on DNA damage and repair in Escherichia coli." J. Antimicrob. Chemother. 1990			
		May;25(5):733-44. Abstract only			

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	16	Tiller G et al. "A recurrent RNA-splicing mutation in the SEDL gene causes X-linked spondyloepiphyseal dysplasia tarda." Am. J. Hum. Genet. 68:1398-1407, 2001.			
	17	SPAYDE E et al. "Exon skipping mutation in the COL9A2 gene in a family with multiple epiphyseal dysplasia." Matrix Biol. 19:121-8 2000. Abstract only			
	18	VAILLY et al. "Identification of a homozygous exon-skipping mutation in the LAMC2 gene in a patient with Herlitz's junctional epidermolysis bullosa."			
		J. Invest. Dermatol. 1995 Mar;104(3):434-7. Abstract only			
	19	WELCH E et al. "PTC124 targets genetic disorders caused by nonsense mutations." Nature 447:87-91, 2007.			
	20	YEOWELL H et al. "Ehlers-Danlos syndrome type VI results from a nonsense mutation and a splice site-mediated exon-skipping mutation in the lysyl hydroxylase gene."			
		Proc. Assoc. Am. Physicians 1997 Jul;109(4):383-96. Abstract only			
	21	Selleck Chemicals, online catalog, PTC124 (Ataluren) 2010			

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